

4 Testing Medicines Scientifically



How can we be certain that the foods we eat are safe, that the cosmetics we use won't harm us, and that medical products are effective? In the United States, the Federal Food, Drug, and Cosmetic Act and several other laws protect the public's safety. Before the Federal Food, Drug, and Cosmetic Act was passed in 1938, most products were not regulated.

CHALLENGE

How are medicines, such as over-the-counter and prescription drugs, tested?

READING

The Role of the FDA

Today, the U.S. Food and Drug Administration (FDA) enforces the laws on product safety and effectiveness. The FDA approves medicines that have been proven to be safe and effective for people to use. To be approved, a product must undergo and pass scientific tests. Usually, new medicines are first tested in animals to see whether they cause any harmful side effects. Only then are the medicines tested by volunteers in clinical trials. The results of clinical trials must show that the medicine is both safe and effective before it can be approved for use by the public.

STOPPING TO THINK 1

What are two reasons that medicines must be tested before they are made available to the public?

Informed Consent

In clinical trials, volunteers are chosen carefully. They must not be allergic to drugs similar to the one that will be tested. Also, they cannot be taking certain other medications. These things might make it more likely for them to experience harmful side effects. The volunteers must be told about any risks, such as possible side effects. They sign an **informed consent** form. This form states that they have been told (informed) about the risks and that they agree (consent) to participate in the trial.

STOPPING TO THINK 2

- a. Why do volunteers in a clinical trial have to sign informed consent forms?
 - b. Make a list of all of the information that you think should be included in an informed consent form.
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Treatment and Control Groups

The volunteers are then divided into two groups. One group receives a pill or liquid that contains the medicine being tested. The other group receives a pill or liquid that looks the same but contains only an inactive ingredient, such as sugar. This inactive pill is called a **placebo** (pla-SEE-bo). Neither the patients nor their doctors know who is receiving the medicine and who is receiving the placebo. The placebo is used as a **control**. It helps to prove that any improvement in the patient is due to the medicine and not to other aspects of the medical treatment. For example, just going to see a doctor may result in an improvement in a patient's condition.



STOPPING TO THINK 3

- a. Explain the relationship between a placebo and a control.
 - b. Why do you think the doctor isn't told whether the patient is receiving the treatment or the placebo? Imagine you are the doctor. How would you act if you thought your patient was getting a treatment that will work? How would you act if you thought your patient was getting just a placebo? How might this affect the patient?
 - c. What is the purpose of a placebo?
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The Placebo Effect

Why is it necessary to use placebos in clinical trials? For many years, scientists observed that people in clinical trials who receive placebos were more likely to show an improvement than other patients with the same disease. This improvement in the health of people receiving placebos is known as the **placebo effect**. Scientists give several explanations for the placebo effect. One factor is the regular, and often better, medical care that patients receive in a clinical trial. Another factor is the psychological effect of participating in a clinical trial. A patient who is getting regular medical attention and a pill may have a more positive attitude toward his or her health. He or she may also be more careful about diet, exercise, or other factors related to the illness. As a result, this person may be more likely to recover because of this positive attitude, and not as a result of anything in the pill. Today, scientists and doctors are studying this connection between a person's mental attitude and his or her physical health to understand how they influence each other.

STOPPING TO THINK 4

In your own words, what is the placebo effect?

The placebo effect sometimes makes it difficult to determine if a treatment is effective. For example, the use of vitamin C to treat colds is controversial. Some studies suggest that vitamin C improves cold symptoms. Other studies suggest that vitamin C doesn't work any better than a placebo, and that people who feel better from vitamin C are experiencing a placebo effect. Studies of high blood pressure, asthma, pain, depression, and cough have shown that about 30–40% of patients taking a placebo experience some relief of their symptoms. This relief can sometimes be measured objectively: for example, blood pressure actually drops in some patients taking placebos.

ANALYSIS

1. In clinical trials of medicines, why is one group of volunteers given a placebo? Explain.
2. Activity 3, "Testing Medicines: A Clinical Trial," simulated a clinical trial that investigated whether a headache medicine was effective. A person reporting a better or worse taste with the pink lemon drink (compared with the yellow lemon drink) represented a person feeling better after taking a pill for headache relief. In this simulation:
 - a. What represented the medicine?
 - b. What represented the placebo?
 - c. Look at your data on Student Sheet 3.1, "Analysis of Clinical Trial." How many people experienced the placebo effect?
 - d. Look at your data on Student Sheet 3.1. How many people in the placebo group were unaffected by the placebo?
3. Imagine a clinical trial to test a treatment for serious illnesses, such as heart disease or cancer. What is the trade-off of giving placebos to some people participating in this clinical trial?



Table 1: Clinical Trial of Cold Medicine				
	Feel the Same	Feel Better	Feel Worse	Total Number of People in Group
Control Group (Received placebo)	60	35	5	100
Treatment Group (Received medicine)	10	80	10	100



4. Review the data shown above from a clinical trial of a cold medicine.
 - a. Copy and complete a table like the one shown below to compare the number of people who feel better as a result of the medicine vs. the placebo:

Table 2: Analysis of Clinical Trial Data				
		Number of people who feel better	Total number of people in group	Percent who feel better
Control group (Received placebo)				
Treatment group (Received medicine)				

- b. Is the medicine effective in a high percentage of the population? Explain your answer.
 - c. Compare the percentage of people who feel worse in the placebo group vs. the percentage who feel worse in the treatment group. What difference do you observe? What could explain this difference?
 - d. Would you conclude that this medicine is safe and effective for treating colds? Explain the evidence for your conclusion.

Table 3: Clinical Trial of Headache Pill

	Feel the Same	Feel Better	Feel Worse	Total Number of People in Group
Control Group (Received placebo)	50	46	4	100
Treatment Group (Received medicine)	41	55	4	100

5. Review the data shown above from a clinical trial of a headache pill. Think about whether the medicine works and how often side effects occur. Explain why this medicine should or should not be sold. Support your answer with evidence about the safety and effectiveness of this medicine.
6. **a. Reflection:** What evidence do you use to decide if cold, headache, or other over-the-counter medicines work for you?
- b.** What effect do you think a positive attitude has on your everyday health? What about when you are sick?